

**Table 1: Current Status of Drugs Which Have Been Listed by NIH (NICHD) for BPCA
As of March 28, 2007**

Drug	Indication	Listing	Patent Status	Written Request/ RFP	Clinical Trial Primary Site	Current Status and/or Clinical Trial Design
13-Cis-Retinoic Acid	Neuroblastoma	2007	Off-patent	Pending	Pending	Pending
*Acyclovir	Herpetic infections	2005	On-patent	FDA	N/A	Being reviewed for re-labeling based on published literature.
Ampicillin	Infection	2004	Off-patent	NICHD	N/A	RFC under development, data gathering in process.
Ampicillin/sulbactam	Infection	2003	Off-patent	FDA	N/A	Inactive.
Azithromycin (IV)	Prevention of bronchopulmonary dysplasia (BPD) in neonates colonized with Ureaplasma urealyticum	2003	Off-patent	NICHD	N/A	Currently reviewing available scientific data on safety and efficacy.
Azithromycin (PO)	Prevention treatment of Chlamydia conjunctivitis and pneumonia	2003	Off-patent	NICHD	N/A	Memo to file finalized that due to feasibility issues and lack of response to RFP, clinical studies could not be done.
*Baclofen	Oral treatment of spasticity from cerebral palsy	2003	On-patent	NICHD	Washington University of St. Louis	Chart review underway. Pharmacokinetics, safety and efficacy studies to be performed.
Bumetanide	Diuresis	2003	Off-patent	FDA	N/A	Memo to file pending that based on scientific recommendation, studies are not recommended at this time.
*Bupropion	Treatment of Depression	2004	On-patent	FNIH NICHD	N/A	Consultation with scientific community completed. Written Request referred to FNIH.
*Bupropion	Treatment for smoking cessation	2004	On-patent	FNIH NICHD	N/A	Written Request referred to FNIH.
Clindamycin	Methicillin-resistant Staphylococcus aureus infection	2007	Off-patent	Pending	Pending	Pending
Clonidine	Autism	2005	Off-patent	FDA	N/A	Inactive.
Clonidine	Attention deficit disorder	2005	Off-patent	FDA	N/A	Preliminary consultation with scientific community completed and currently reviewing scientific information from a previously performed clinical study.
Cyclosporine	Cardiac transplant rejection	2005	Off-patent	FDA	N/A	Inactive.

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D-actinomycin	Cancer	2004	Off-patent	NICHHD	NICHHD Partnership with NCI/COG	NCI/COG studies underway.
Daunomycin	Cancer	2006	Off-patent	NICHHD	NICHHD Partnership with NCI/COG	NCI/COG studies underway.
*Dexrazoxane	Prophylaxis from cardiotoxicity of doxorubicin	2005	On-patent	FNIH NICHHD	N/A	WR referred to FNIH.
Diazoxide	Hypoglycemia	2003	Off-patent	FDA	N/A	Memo to file pending that due feasibility issues, studies not recommended
Dobutamine	Hypotension, low cardiac output in neonates	2003	Off-patent	FDA	N/A	Memo to file pending that due to issues with feasibility and clinical trial design, studies not recommended at this time.
Dopamine	Hypotension, low cardiac output in neonates	2003	Off-patent	FDA	N/A	Memo to file pending that due to issues with feasibility and clinical trial design, studies not recommended at this time.
Doxycycline	Methicillin-resistant Staphylococcus aureus infection	2007	Off-patent	Pending	Pending	Pending
*Eletriptan	Migraine headaches in adolescents	2005	On-patent	FNIH NICHHD	N/A	WR referred to FNIH.
Ethambutol	Tuberculosis	2005	Off-patent	FDA	N/A	Data gathering in process.
Flecainide	Ventricular arrhythmia	2005	Off-patent	FDA	N/A	Data gathering in process.
Furosemide	Diuresis	2003	Off-patent	FDA	N/A	Memo to file pending that based on scientific recommendation, studies are not recommended at this time.
Griseofulvin	Tinea capitis	2005	Off-patent	NICHHD	N/A	RFC under development, data gathering in process.
Heparin	Anticoagulation	2003	Off-patent	FDA		Already labeled for patients ≥ 1 kg. Memo to file finalized that no further study recommended.

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Hydrochlorothiazide	Hypertension	2005	Off-patent	FDA	N/A	Consultation with scientific community concerning diagnosis and treatment of pediatric hypertension completed and recommendations provided to NICHD/FDA. WR in process.
*Hydrocortisone valerate ointment and cream	Dermatitis	2005	On-patent	FDA	N/A	Inactive.
Hydroxychloroquine	Connective tissue disorders	2005	Off-patent	FDA	N/A	Literature review in process.
*Hydroxyurea	Sickle Cell Disease	2006	On-patent	FNIH NICHD	NICHD Partnership with NHLBI	PK, efficacy and safety studies continue. Participants currently being enrolled.
Isoflurane	Maintenance of general anesthesia	2003	Off-patent	FDA	N/A	Awaiting results of Ketamine preclinical study.
Ivermectin	Scabies	2005	Off-patent	FDA	N/A	Memo to file finalized that formulation issues preclude study.
Ketamine	Sedation	2004	Off-patent	FDA	NICHD Partnership with NCTR, FDA	Pre-clinical toxicology studies underway, results pending.
Lindane	Second line treatment of scabies	2003	Off-patent	FDA	N/A	WR accepted by NDA holder. Results pending.
Lithium	Treatment of mania in bipolar disorder	2003	Off-patent	NICHD	Case Western Reserve University	PK, safety, efficacy, and tolerability studies underway. Participants currently being enrolled.
Lorazepam	Treatment of Status Epilepticus	2003	Off-patent	NICHD	Children's National Medical Center	PK study complete. Follow up study pending community consultation.
Lorazepam	Sedation in the intensive care unit for children on respirators	2003	Off-patent	NICHD	Case Western Reserve University	Pharmacokinetics, safety and efficacy studies continue. Participants are currently being enrolled.
Meropenem	Infection	2003	Off-patent	NICHD	N/A	Currently in contract negotiations.
Methadone	Neonates with opioid withdrawal	2005	Off-patent	FDA	N/A	Data gathering in process.
Methotrexate	Cancer	2006	Off-patent	NICHD	NICHD Partnership with NCI/COG	NCI/COG studies underway.
*Metoclopramide	Gastro-esophageal reflux	2003	On-patent	FNIH NICHD	N/A	WR referred to FNIH.

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*Methylphenidate	Attention Deficit and Hyperactivity Disorder	2005	On-patent	NICHHD	NICHHD Partnership with NIEHS, NIMH, FDA/NCTR	Currently under evaluation of genetic toxicity based on published report of cytogenetic effect of Methylphenidate reported in <u>Cancer Letters</u> (2005).
Metolazone	Diuresis	2004	Off-patent	FDA	N/A	Memo to file pending that based on scientific recommendation, studies are not recommended at this time.
*Morphine	Analgesia	2004	On-patent	FNIH NICHHD	Children's National Medical Center	Grant awarded by NICHHD in 2005 for study in neonates and is currently underway.
Piperacillin/tazobactam	Infection	2003	Off-patent	FDA	N/A	Already labeled down to 2 months of age. Memo to file pending that no further study recommended.
Pralidoxime	Organophosphate Poisoning	2006	Off-patent	FDA	N/A	Systematic literature review underway for potential label update.
Promethazine	Nausea and vomiting	2003	Off-patent	FDA	N/A	Currently has black boxed warning. Memo to file pending.
Rifampin	Methicillin- resistant Staphylococcus aureus endocarditis	2003	Off-patent	NICHHD	N/A	Inactive. Frequency of condition being reviewed.
Rifampin	Central nervous system shunt infection	2003	Off-patent	NICHHD	N/A	Inactive. Frequency of condition being reviewed.
*Sevelamer	Hyperphosphatemia in chronic renal failure	2005	On-patent	FNIH NICHHD	N/A	Written Request referred to FNIH.
Sodium nitroprusside	Control of blood pressure	2003	Off-patent	NICHHD	Duke and Stanford Universities	Pharmacokinetics, safety, studies underway. Participants continue to be enrolled.
Spironolactone	Diuresis	2003	Off-patent	FDA	N/A	Memo to file pending that based on scientific recommendation, studies are not recommended at this time.
Tetracycline	Methicillin-resistant Staphylococcus aureus infection	2007	Off-patent	Pending	Pending	Pending
*Trimethoprim-Sulfamethoxazole	Methicillin-resistant Staphylococcus aureus infection	2007	On-patent	Pending	Pending	Pending
Vincristine	Cancer	2004	Off-patent	NICHHD	NICHHD Partnership with NCI/COG	NCI/COG studies underway.

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*Zonisamide	Partial Seizures	2005	On-patent	FNIH NICHD	N/A	Written Request referred to FNIH.

Key: Drug is the generic name, Indication summarizes the indication or condition for which the drug is to be tested, Listing notes the year in which the drug was added to the list for testing, Patent Status is the on or off patent status of the drug, WR indicates a Written Request has been issued by the FDA and denotes where the request for and the processing of information currently resides {FDA, Foundation for NIH (FNIH) or NIH}, Request for Proposals (RFP) indicates the RFP was published by NICHD and its current status, Clinical Trial Primary Site identifies the institution who has received the contract and has designed and implemented the pre-clinical or clinical protocol, Clinical Trial Design indicates in general terms the format of the proposed or actual pre-clinical or clinical trials and the phases of drug development being conducted. N/A refers to a process that is not applicable to a particular drug at this time. Inactive generally refers to feasibility issues that make the formation of the WR or RFP difficult to complete.

NHLBI is the National Heart, Lung, and Blood Institute, NCI is the National Cancer Institute, COG is the Children's Oncology Group, NIEHS is the National Institute of Environmental Health Sciences, NIMH is the National Institute of Mental Health, NCTR is the National Center for Toxicology Research.

*Indicates that a drug is currently on-patent and will be studied under a different funding mechanism than the off-patent process as described in the BPCA Legislation of 2002. For an on-patent drug, if the manufacturer has denied or failed to respond to the WR issued by the FDA in 120 days, the FDA refers the drug to the FNIH and requests that it be considered for FNIH support of pediatric studies. These drugs are also discussed at the annual scientific listing meetings.